

Stem Cell Agency Approves Funding for Clinical Trials Targeting Parkinson's Disease and Blindness

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Oakland, CA – The governing Board of the California Institute for Regenerative Medicine (CIRM) today invested \$32.92 million to fund the Stem Cell Agency's first clinical trial in Parkinson's disease (PD), and to support three clinical trials targeting different forms of vision loss.

This brings the total number of clinical trials funded by CIRM to 60.

The PD trial will be carried out by Dr. Krystof Bankiewicz at Brain Neurotherapy Bio, Inc. He is using a gene therapy approach to promote the production of a protein called GDNF, which is best known for its ability to protect dopaminergic neurons, the kind of cell damaged by Parkinson's. The approach seeks to increase dopamine production in the brain, alleviating PD symptoms and potentially slowing down the disease progress.

David Higgins, PhD, a CIRM Board member and patient advocate for Parkinson's says there is a real need for new approaches to treating the disease. In the US alone, approximately 60,000 people are diagnosed with PD each year and it is expected that almost one million people will be living with the disease by 2020.

"Parkinson's Disease is a serious unmet medical need and, for reasons we don't fully understand, its prevalence is increasing. There's always more outstanding research to fund than there is money to fund it. The GDNF approach represents one 'class' of potential therapies for Parkinson's Disease and has the potential to address issues that are even broader than this specific therapy alone."

The Board also approved funding for two clinical trials targeting retinitis pigmentosa (RP), a blinding eye disease that affects approximately 150,000 individuals in the US and 1.5 million people around the world. It is caused by the destruction of light-sensing cells in the back of the eye known as photoreceptors. This leads to gradual vision loss and eventually blindness. There are currently no effective treatments for RP.

Dr. Henry Klassen and his team at jCyte are injecting human retinal progenitor cells (hRPCs), into the vitreous cavity, a gel-filled space located in between the front and back part of the eye. The proposed mechanism of action is that hRPCs secrete neurotrophic factors that preserve, protect and even reactivate the photoreceptors, reversing the course of the disease.

CIRM has supported early development of Dr. Klassen's approach as well as preclinical studies and two previous clinical trials. The US Food and Drug Administration (FDA) has granted jCyte Regenerative Medicine Advanced Therapy (RMAT) designation based on the early clinical data for this severe unmet medical need, thus making the program eligible for expedited review and approval.

The other project targeting RP is led by Dr. Clive Svendsen from the Cedars-Sinai Regenerative Medicine Institute. In this approach, human neural progenitor cells (hNPCs) are transplanted to the back of the eye of RP patients. The goal is that the transplanted hNPCs will integrate and create a protective layer of cells that prevent destruction of the adjacent photoreceptors.

The third trial focused on vision destroying diseases is led by Dr. Sophie Deng at the University of California Los Angeles (UCLA). Dr. Deng's clinical trial addresses blinding corneal disease by targeting limbal stem cell deficiency (LSCD). Under healthy conditions, limbal stem cells (LSCs) continuously regenerate the cornea, the clear front surface of the eye that refracts light entering the eye and is responsible for the majority of the optical power. Without adequate limbal cells, inflammation, scarring, eye pain, loss of corneal clarity and gradual vision loss can occur. Dr. Deng's team will expand the patient's own remaining LSCs for transplantation and will use novel diagnostic methods to assess the severity of LSCD and patient responses to treatment. This clinical trial builds upon previous CIRM-funded work, which includes early translational and late stage preclinical projects.

"CIRM funds and accelerates promising early stage research, through development and to clinical trials," says Maria T. Millan, MD, President and CEO of CIRM. "Programs, such as those funded today, that were novel stem cell or gene therapy approaches addressing a small number of patients, often have difficulty attracting early investment and funding. CIRM's role is to de-risk these novel regenerative medicine approaches that are based on rigorous science and have the potential to address unmet medical needs. By de-risking programs, CIRM has enabled our portfolio programs to gain significant downstream industry funding and partnership."

CIRM Board also awarded \$5.53 million to Dr. Rosa Bacchetta at Stanford to complete work necessary to conduct a clinical trial for IPEX syndrome, a rare disease caused by mutations in the FOXP3 gene. Immune cells called regulatory T Cells normally function to protect tissues from damage but in patients with IPEX syndrome, lack of functional Tregs render the body's own tissues and organs to autoimmune attack that could be fatal in early childhood. Current treatment options include a bone marrow transplant which is limited by available donors and graft versus host disease and immune suppressive drugs that are only partially effective. Dr. Rosa Bacchetta and her team at Stanford will use gene therapy to insert a normal version of the FOXP3 gene into the patient's own T Cells to restore the normal function of regulatory T Cells.

The CIRM Board also approved investing \$15.80 million in four awards in the Translational Research program. The goal of this program is to help promising projects complete the testing needed to begin talking to the US Food and Drug Administration (FDA) about holding a clinical trial.

The TRAN1 Awards are summarized in the table below:

Application	Title	Institution	Award Amount
TRAN1 – 11536	<i>Ex Vivo Gene Editing of Human Hematopoietic Stem Cells for the Treatment of X-Linked Hyper IgM Syndrome</i>	UCLA	\$4,896,628
TRAN1 – 11555	<i>BCMA/CS1 Bispecific CAR-T Cell Therapy to Prevent Antigen Escape in Multiple Myeloma</i>	UCLA	\$3,176,805
TRAN1 – 11544	<i>Neural Stem cell-mediated oncolytic immunotherapy for ovarian cancer</i>	City of Hope	\$2,873,262
TRAN1 - 11611	<i>Development of a human stem cell-derived inhibitory neuron therapeutic for the treatment of chronic focal epilepsy</i>	Neurona Therapeutics	\$4,848,750

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet

medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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